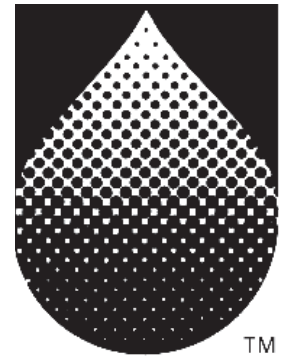


Aplastic Anemia & MDS International Foundation, Inc.

Fighting Bone Marrow Diseases through Patient Support & Research Since 1983



Patient Information

Our expert Medical Board will answer your questions and provide information on any issue. Call us – we are standing by ready to help you!



Ask the Experts

Go to www.aamds.org to review the archive of patients' questions which have been answered by our distinguished panel of medical experts. You can also personally ask your own question of these experts at this site.



Patient Clinical Trials

We give you an updated list featuring doctor's name, phone number and explanation of treatment protocols.



Patient Travel Fund

Families traveling to clinical trials can receive up to \$500 for travel expenses. Contact our office to receive an application form.



Global Network of Volunteers

You are not alone – patients and families will share their treatment experiences with you. Call us to make a friend!



Patient Registry

Statistics on these diseases seem to be as rare as the diseases themselves. Please fill out our Patient Registry Form for surviving or deceased patients. The more data we collect, the more data we can use for research.



You Can Help

Please donate your blood & platelets, have your bone marrow tested, and financially donate to the AA&MDSIF's research & support efforts.

Oral Iron Chelator Exjade® Approved by FDA

The AA&MDSIF applauds the Food and Drug Administration's (FDA's) approval of Exjade® (ICL 670) manufactured by Novartis, Inc., a once daily oral iron chelator for patients (age two and older) with transfusion based iron overload. Exjade® is administered as a drink. The tablets are dissolved in a glass of orange juice, apple juice, or water.

"The FDA's approval of Exjade® provides our transfusion-dependent patients with a welcome new treatment option for the potentially fatal effects caused by iron overload," said Robert Carroll, AA&MDSIF president and transfusion-dependent patient for 17 years.

The current standard of care Desferal® (Novartis, Inc.), which often requires a subcutaneous infusion lasting eight to 12 hours per night, for five to seven nights a week for as long as the patient continues to receive blood transfusions or has excess iron within the body. As a result of this regime, many patients may have stopped or avoided iron chelation therapy, thus risking the toxic effects of iron overload.

To learn more about Exjade®, patients and physicians in the U.S. can call Novartis, Inc. at 888.669.6682. To learn more about ongoing Exjade clinical trials, health care providers can call either 800.340.6843 in the U.S., or +44 (0) 1506 814899 outside the U.S. ♦

Website Redesigned to Better Serve Patients

Thanks to an unrestricted educational grant from Pharmion Corporation (makers of Vidaza) we have updated our website: www.aamds.org.

After listening to your suggestions, we carefully redesigned our website to help you get the information you need. Check out our DRUG INFORMATION area to read about your medications. Review our archived QUESTIONS FOR THE EXPERTS area to see how the experts answer your disease inquiries. Go to the INFORMATION AND HOPE area to see what you missed in past issues of this newsletter and ebulletins. And make sure you survey PATIENT CLINICAL TRIALS section to discover the newest research studies. ♦

Drug Updates

Research is continually ongoing in bone marrow diseases in order to find the best treatment options. Following are updates on several drugs that are showing promise in the treatment of these diseases and nearing approval by the Food and Drug Administration - Revlimid®, Dacogen®, and Eculizumab®. For continually updated information please visit our website or call us to speak with one of our medical librarians.

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From the Director...

There is one thing even more vital to science than intelligent methods; and that is the sincere desire to find out the truth, whatever it may be.
-Charles Sanders Pierce



Looking around the room at our recent International Bone Marrow Scientific Symposium I was awestruck at the intellect we had gathered together. The world's most distinguished leading experts in bone marrow diseases eagerly presented their work to over one hundred doctors from around the globe. Doctors from Thailand were proposing ideas to the Italian researchers, doctors from the Netherlands were discussing issues with researchers from France, and American doctors were analyzing causes with researchers from Japan. Everyone in the room had dedicated their career to bone marrow diseases...everyone was sharing knowledge to find the cure...everyone was working hard to help their patients. With such great minds collaborating, we have reason to hope!

And, I marveled at how far we had come. Decades ago when the AA&MDSIF was first organized, this type of meeting couldn't even be considered because there just wasn't enough interest in such rare diseases. Now, in 2005, 150 researchers were enthusiastically comparing scientific data and discussing ideas late into the night. I couldn't help but think that the spirits of thousands of patients were with us during this meeting of the minds urging us to work even harder.

This important scientific meeting is just one of the projects the AA&MDSIF is able to fund thanks to your donations. Your contributions also fund vital medical research to find better treatments, help patients and their families learn about treatment options, and spread awareness of the importance of curing these rare diseases.

If you agree that our work is valuable in truly helping patients and finding a cure, then we ask you to please be generous when you receive our end-of-year appeal letter. ♦

*-Marilyn Baker, M.S.
Executive Director & Editor*

Efficiency with your donations is our strength...

At AA&MDSIF, 90.4% of your donation goes directly to medical research, patient support, education and advocacy.

Unfortunately there was a typo in our year-end appeal. We meant to say that we spend far less on fund-raising than the national average. **And we spend FAR MORE than the national average on critical patient support and medical research.**

The American Institute of Philanthropy has determined the reasonable industry standard for administrative and fundraising expenses to be 40%. **AA&MDSIF is proud to only spend 9.6%** on fundraising and administrative expenses. We strongly encourage donors to compare our performance to other organizations when making charitable contributions.

We continue to dedicate ourselves to maintaining this low annual percentage in order to allow maximum resources to be passed on to patient information and research.

Patient Organizers

This three-ringed binder with pre-printed tabs is an excellent tool to keep all medical information in one place convenient for phone calls and doctor visits. Organize important medical contacts, medications list, transfusion history, lab reports, lifestyle guidelines, etc. in this handy notebook. Thanks to Pharmion Corp., makers of Vidza®, these notebooks are FREE to all patients. Place your order via website or phone.

Bravery Bracelets

Show your support! These red & white bracelets (symbolizing red & white blood cells) have the word "Bravery" etched in them as a reminder of the daily courage our patients and loved ones have to battle bone marrow disease. Help spread awareness of bone marrow diseases! Ask friends and family to wear the band that helps fight disease – a great little holiday giveaway! Place your bulk order today via website or phone.

Conference CD

Hear the Experts! For those who couldn't attend this year's Patient and Family Conference, we have CD's available for a \$30 donation. Hear presentations from Dr. Marlene Haffner, Dr. Mikkael Sekeres, Dr. Pam Becker, Dr. Richard Childs, Dr. Lewis Silverman, and Dr. Charles Parker. Place your order via website or phone.

Scientific Symposium Report

Read the Experts! We have written a Scientific Symposium Report summarizing our recent International meeting (see story on page 4) Thanks to an unrestricted educational grant from Novartis Inc., makers of Exjade®, this report is FREE to all patients. Order the absolute most up-to-date reporting on bone marrow failure diseases from the world's leading experts via website or phone. ♦

Working for the Cause

\$103,000 for the Pursuing New Hope Research Award

THANK YOU to everyone who answered our matching gift challenge in the Spring 2005 Newsletter. Your generous donations to the Pursuing New Hope Award totaled \$39,000! Our anonymous donor has matched your contributions enabling us to give \$103,000 to fund medical research investing effective treatments and cure for paroxysmal hemoglobinuria (PNH). While a single donation might not help find a cure, combining our contributions together has created a dynamic research study. Your support made this research happen! Thank you!



Sri Lanka Minister of Health, Robert Carroll, Prime Minister Mahinda Rajapaksa and other dignitaries

AA&MDSIF President in Sri Lanka

Robert Carroll recently traveled to Sri Lanka and met with the Minister of Health and Prime Minister Mahinda Rajapaksa. He made the long journey to discuss the treatment of MDS and education of patients in their country. Bob arranged for the AA&MDSIF to provide materials to the country's hospitals and clinics. Our thanks to Bob for giving aid to a country ravaged by the Tsunami and bone marrow disease.

The Earl J. Goldberg Legacy

In 1979, 14-year-old Earl was diagnosed with aplastic anemia which at that time, was not treatable, and within three months he passed away. Friends of his parents, Harriet and Alan, organized themselves into America's first bone marrow foundation. They created the very first pamphlets on aplastic anemia and were the first to begin funding research. And in 1983, when the A&MDSIF was just getting started, the Earl J. Goldberg Foundation generously gave their time and money to help us get organized. During the past decades we worked closely together struggling to put an end to this disease. When Marilyn Baker first started as the AA&MDSIF director in 1990, Harriet trained her with an iron-fisted mentorship that continues to this day. Now, after 26 years of helping patients, raising awareness, and donating nearly \$200,000 to various AA&MDSIF projects, they have closed their doors. While their efforts were a tribute to Earl, what they created was a loving legacy of friendship...to Harriet and Alan Goldberg.

East Meets West

Ginny Gong, host of the television series "East Meets West", invited Dr. Yong Tang, bone marrow disease researcher from the National Heart, Lung & Blood Institute and Neil Horikoshi, AA&MDSIF board member, to come on her program to discuss the high incidence of aplastic anemia in Asians. We are grateful to Ginny for helping the AA&MDSIF raise awareness of this issue. ♣



Ginny Gong interviews Neil Horikoshi

International Bone Marrow Disease Scientific Symposium

The Aplastic Anemia & MDS International Foundation was proud to bring the world's leading medical researchers to Washington DC for a two-day meeting in October to discuss the latest research findings for bone marrow diseases.

Nearly 150 physicians from 17 different countries with interests in immunosuppressive therapy, stem cell transplantation and basic scientists in cell biology, and immunology education gathered together and exchanged ideas relating to the etiology, genetics, pathophysiology, epidemiology, and treatment of bone marrow failure diseases.

Co-chairs **Dr. Jaroslaw Maciejewski** of the Taussig Cancer Center Cleveland Clinic and **Dr. Neal Young** of the National Heart, Lung, and Blood Institute presided over this prestigious meeting featuring the following presenters:

~

Dr. A. John Barrett, National Heart, Lung and Blood Institute, National Institutes of Health, Maryland

Dr. Theo de Witte, Radboud University Nijmegen Medical Centre Central, The Netherlands;

Dr. Elihu Estey, University of Texas MD Anderson Cancer Center, Texas;

Dr. Rupert Handgretinger, St. Jude Children's Research Hospital, Tennessee;

Dr. Peter Hillmen, Leeds Teaching Hospital, United Kingdom;

Dr. Surapol Issaragrisil, Siriraj Hospital, Mahidol University, Thailand;

Dr. David Kaufman, Boston University School of Public Health, Massachusetts;

Dr. Seiji Kojima, Nagoya University Graduate School of Medicine, Japan;

Dr. Alan List, H. Lee Moffitt Cancer Center & Research Institute, Florida;

Dr. Lucio Luzzatto, Instituto Toscazo Tumori Firenze & University of Genova, Italy

Dr. David Margolis, Children's Hospital of Wisconsin, Wisconsin;

Dr. Judith Marsh, St. Georges Hospital Medical School, University of London, United Kingdom;

Dr. Jeffrey Molldrem, University of Texas MD Anderson Cancer Center, Texas;

Dr. Shinji Nakao, Kanazawa University of Graduate School of Medical Science, Japan;

Dr. Elaine Sloand, National Heart, Lung, and Blood Institute, National Institutes of Health, Maryland;

Dr. Gerard Socie, Universite Paris VII & Hopital Saint Louis, France;

Dr. Andre Tichelli, University Clinics, Switzerland ♦

A report on this meeting is available free of charge, thanks to an unrestricted educational grant from Novartis, Inc.



Dr. Kaufman, Dr. Issaragrisil, Neil Horikoshi, Dr. Kojima, Dr. Young, Dr. Nakao discuss disease incidence in Asians



Dr. Handgretinger presenting his research findings



P.K. Burnette and Dr. List join in discussions



Dr. Luzzatto and Dr. Hillmen discuss research

Revlimid® update

The new action date for Revlimid's® (lenalidomide) New Drug Application (NDA) by the Food and Drug Administration (FDA) is set for January 7, 2006. The extension is a result of the FDA requiring more time to review additional existing information on Celgene's risk-management program. On October 26, 2005 The European Medicines Agency (EMA) accepted Celgene's Marketing Authorization Application. Revlimid® is a treatment for transfusion dependent anemia due to low-or-intermediate-risk myelodysplastic syndromes (MDS) associated with the cytogenetic abnormality deletion 5q.

Dacogen® update

MGI Pharma and SuperGen announced on September 13, 2005 that they received an approvable letter from the U.S. Food and Drug Administration (FDA) for Dacogen® (decitabine) injection for the treatment of myelodysplastic syndromes (MDS). This letter from the FDA requested additional information, including analysis of the transfusion requirements of patients enrolled in the completed phase three clinical trial, and submission of other information.

Eculizumab® update

Alexion Pharmaceuticals, Inc. announced on September 21, 2005 that the Phase III trial of Eculizumab® for patients with PNH has begun. Eculizumab® is in a class of drugs called monoclonal antibodies. This drug blocks the effects of the complement system (part of the immune system) on PNH blood cells and stops hemolysis, the destruction of blood cells. This trial is scheduled to last for twelve months with a six month analysis. ♦

Recent Medical Literature

Listed below are recently published journal articles indexed in the National Library of Medicine database MEDLINE: www.pubmed.gov. To obtain complete articles, contact your public or hospital library. These articles are listed for general information purposes only.

Aplastic Anemia

Fuhrer M, Rampf U, Baumann I, et al. Immunosuppressive therapy for aplastic anemia in children: a more severe disease predicts better survival. *Blood*. 2005 Sep 15;106(6):2102-4.

Issaragrisil S, Kaufman DW, Anderson T, et al. The epidemiology of aplastic anemia in Thailand. *Blood*. 2005 Oct 27; [Epub ahead of print].

Kurre P, Johnson FL, Deeg HJ. Diagnosis and treatment of children with aplastic anemia. *Pediatric Blood Cancer*. 2005 Nov; 45(6):770-80.

Nakao S, Feng X, Sugimori C. Immune pathophysiology of aplastic anemia. *International Journal of Hematology*. 2005 Oct;82(3):196-200.

Sloand E. Genetic polymorphisms and the risk of acquired idiopathic aplastic anemia. *Haematologic*. 2005 Aug; 90(8):1009B.

Myelodysplastic Syndromes

Bennett JM, Komrokji RS. The Myelodysplastic syndromes: Diagnosis, molecular biology and risk assessment. *Hematology*. 2005 Sep-Oct; 10 Suppl 1:258-69.

Giagounidis AA, Germing U, Strupp C, et al. Prognosis of patients with del(5q) MDS and complex karyotype and the possible role of lenalidomide in this patient subgroup. *Annals of Hematology*. 2005 Sep;84(9):569-71. Epub 2005 May 13.

Hamblin TJ. The management of anemia in the myelodysplastic syndromes. *Leukemia Research*. 2005 Oct; 29(10):1101-2. [Epub 2005 Apr 9].

Lindberg EH. Strategies for biology- and molecular-based treatment of myelodysplastic syndromes. *Current Drug Targets*. 2005 Sep; 6(6):713-25.

Manabe A, Kojima S. 11th symposium on aplastic anemia and 9th symposium on myelodysplastic syndromes in children. *Pediatrics International*. 2005 Oct;47(5):572-4.

Strom SS, Gu Y, Gruschkus SK, et al. Risk factors of myelodysplastic syndromes: a case-control study. *Leukemia*. 2005 Sep 15; [Epub ahead of print].

Paroxysmal Nocturnal Hemoglobinuria

Hill A, Hillmen P, Richards SJ, et al. Sustained response and long-term safety of eculizumab in paroxysmal nocturnal hemoglobinuria. *Blood*. 2005 Oct 1; 106(7):2559-65.

Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Blood*. 2005 Jul 28; [Epub ahead of print].

Pure Red Cell Aplasia

Bennett CL, Cournoyer D, Carson KR, et al. Long-term outcome of individuals with pure red cell aplasia and anti-erythropoietin antibodies in patients treated with recombinant epoetin. *Blood*. 2005 Aug 11; [Epub ahead of print]. ♦

Our sincere gratitude to the Herman Goldman Foundation of New York City for sponsoring this newsletter. Their generous donation has made it possible for us to distribute this newsletter to tens of thousands of patients and their families.

Grassroots Lobbying Secures Support for Bone Marrow Failure Disease Resolution

Thanks to the efforts of our patients, families and friends nationwide, the Aplastic Anemia & MDS International Foundation has secured the support of 35 cosponsors to H.Con.Res. 179, the "Bone Marrow Failure Disease" Resolution. These cosponsors come from the entire liberal to conservative political spectrum. The resolution was introduced at the request of the Foundation by U.S. Representatives Sherrod Brown (D-OH) and Michael Bilirakis (R-FL).

Our next step is to encourage our Congressional allies to move this resolution through the Energy and Commerce Committee and toward passage on the floor of the House of Representatives. If enacted, this resolution would encourage the federal government to fund research and public health initiatives that will lead to improved treatments and new cures for Aplastic Anemia, MDS and PNH.

Since returning in September from its summer recess, Congress has been fully engaged in the recovery efforts for Hurricanes Katrina and Rita, and these activities have postponed the normal business of Congress. According to Energy and Commerce Committee staff, any measures unrelated to the hurricanes will not come before the committee until the committee has had an opportunity to consider a package of budgetary savings to help pay for the recovery efforts.

If you have not already contacted your U.S. Representative to urge them to cosponsor H.Con.Res. 179, please do so today. It is not too late to add cosponsors to this resolution. If you do not know who your Representative is, or need contact information, please access www.house.gov and enter your zip code under the heading "Find Your Representative."

The following U.S. Representatives have cosponsored H.Con.Res. 179:

Abercrombie (HI), Bilirakis (FL), Bishop (NY), Brown (OH), Case (HI), Chocoma (IN), DeFazio (OR), Faleomavaega (American Samoa), Frank (MA), Gillmor (OH), Goode (VA), Gordon (TN), Hinchey (NY), Honda (CA), Johnson (CT), Marshall (GA), Matsui (CA), McDermott (WA), McGovern (MA), McNulty (NY), Moore (KS), Murtha (PA), Oxley (OH), Platts (PA), Rothman (NJ), Schakowsky (IL), Schiff (CA), Schwartz (PA), Shaw (FL), Simmons (CT), Tierney (MA), Van Hollen (MD), Waxman (CA), Wexler (FL), Wolf (VA). ♦



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